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AB Science granted authorization to initiate phase 3 confirmatory study of masitinib in indolent systemic mastocytosis

Company to host live webcast on November 20, 2019 on indolent systemic mastocytosis with Key Opinion Leaders

AB Science SA (NYSE Euronext - FR0010557264 - AB) today announces that it has been authorized by the French Medicine Agency, ANSM, to initiate the Phase 3 confirmatory study evaluating masitinib in indolent systemic mastocytosis.

This study (AB15003) is a multicenter, randomized, double blind, placebo-controlled, phase 3 study to compare the efficacy and safety of masitinib dose titration up to 6 mg/kg/day with that of placebo in treatment of patients with severe indolent systemic mastocytosis, unresponsive to optimal symptomatic treatment.

The study is designed to enroll 140 patients with or without the D816V mutation of c-Kit. The primary endpoint is a measure of the cumulative response on 3 severe symptoms of mast cell mediator release (pruritus, flush and depression) from week 8 to week 24. Secondary endpoints will measure response on the severe symptoms of pruritus, flushes, depression, and fatigue, taken together and individually, quality of life, as well as biological (tryptase) and skin involvement parameters. Under this protocol, severe symptoms of mast cell mediator release (also referred to as handicaps) are defined as: pruritus (score ≥ 9), flush (score ≥ 8), depression (HAMD-17 ≥ 19), and fatigue (FIS ≥ 75 or FSS ≥ 36).

Alain Moussy, CEO and co-founder of AB Science said *“We are committed to bring masitinib as a first therapeutic option for patients suffering from indolent systemic mastocytosis, and we are extremely pleased with this authorization to initiate phase 3, which is a key step in the development program of masitinib in this indication”*.

“Results from our prior phase 3 study, which enrolled 135 patients and was published in The Lancet, showed that masitinib can substantially reduce severe symptoms associated with indolent systemic mastocytosis, regardless of a patient’s c-Kit mutational status,” said Professor Olivier Hermine M.D., President of the Scientific Committee of AB Science and coordinator of the Reference Center for Mastocytosis (CeReMast, Paris, France). *“Moreover, both efficacy and safety data indicate a possibility for the effective long-term management of this difficult-to-treat condition with masitinib. This observation is important, given that indolent systemic mastocytosis is a chronic condition that requires lifelong management”*.

Masitinib’s anti-mast cell properties appear particularly well-adapted to the treatment of indolent systemic mastocytosis. A reduction of mast cell activity is generated via its inhibitory action on wild-type c-Kit, Lyn and Fyn tyrosine kinases. It is through this multifaceted mechanism of action, a feature not observed with other c-Kit inhibitors, that masitinib can elicit a response in patients of both positive and negative D816V c-Kit mutation status.

A webcast call will be organized on 20 November 2019 with the management of AB Science and renowned mastocytosis experts to present in detail the disease, the clinical development landscape, and the results and design of the masitinib program in indolent systemic mastocytosis.

About Indolent Systemic Mastocytosis

Indolent systemic mastocytosis (ISM) is a hematological disease characterized by an abnormal number and activation of mast cells in the bone marrow and other organs. The disease is characterized by multiple symptoms that are disabling and can in some cases be life-threatening. Symptoms associated with ISM are

predominantly associated with neurological disorders (depression, fatigue, cognitive impairment, headache), skin disorders (pruritus, skin lesions), flushing and gastro-intestinal disorders. ISM affects approximately 40,000 people in Europe and 25,000 in the USA. There is currently no therapy approved for the treatment of ISM.

Orphan Drug Status and IP status

Masitinib has been granted orphan drug status in mastocytosis both the FDA and the EMA. The use of masitinib in ISM is covered by patent protection until 2031 in the USA (patent issued) and until 2036 outside of the USA (patent under review).

Prior publications related to masitinib in ISM

- 1) Lancet 2017. *Masitinib for treatment of severely symptomatic indolent systemic mastocytosis: a randomised, placebo-controlled, phase 3 study.* Lortholary O, et al. DOI:[http://dx.doi.org/10.1016/S0140-6736\(16\)31403-9](http://dx.doi.org/10.1016/S0140-6736(16)31403-9),
- 2) PLoS One. 2011. *Depression in patients with mastocytosis: prevalence, features and effects of masitinib therapy.* Moura DS, et al. doi: 10.1371/journal.pone.0026375. Epub 2011 Oct 21.
- 3) Am J Hematol. 2010. *Masitinib for the treatment of systemic and cutaneous mastocytosis with handicap: a phase 2a study.* Paul C, et al. doi: 10.1002/ajh.21894.

About masitinib

Masitinib is a new orally administered tyrosine kinase inhibitor that targets mast cells and macrophages, important cells for immunity, through inhibiting a limited number of kinases. Based on its unique mechanism of action, masitinib can be developed in a large number of conditions in oncology, in inflammatory diseases, and in certain diseases of the central nervous system. In oncology due to its immunotherapy effect, masitinib can have an effect on survival, alone or in combination with chemotherapy. Through its activity on mast cells and microglia and consequently the inhibition of the activation of the inflammatory process, masitinib can have an effect on the symptoms associated with some inflammatory and central nervous system diseases and the degeneration of these diseases.

About AB Science

Founded in 2001, AB Science is a pharmaceutical company specializing in the research, development and commercialization of protein kinase inhibitors (PKIs), a class of targeted proteins whose action are key in signaling pathways within cells. Our programs target only diseases with high unmet medical needs, often lethal with short term survival or rare or refractory to previous line of treatment.

AB Science has developed a proprietary portfolio of molecules and the Company's lead compound, masitinib, has already been registered for veterinary medicine and is developed in human medicine in oncology, neurological diseases, and inflammatory diseases. The company is headquartered in Paris, France, and listed on Euronext Paris (ticker: AB).

Further information is available on AB Science's website: www.ab-science.com.

Forward-looking Statements - AB Science

This press release contains forward-looking statements. These statements are not historical facts. These statements include projections and estimates as well as the assumptions on which they are based, statements based on projects, objectives, intentions and expectations regarding financial results, events, operations, future services, product development and their potential or future performance.

These forward-looking statements can often be identified by the words "expect", "anticipate", "believe", "intend", "estimate" or "plan" as well as other similar terms. While AB Science believes these forward-looking statements are reasonable, investors are cautioned that these forward-looking statements are subject to numerous risks and uncertainties that are difficult to predict and generally beyond the control of AB Science and which may imply that results and actual events significantly differ from those expressed, induced or anticipated in the forward-looking information and statements. These risks and uncertainties include the uncertainties related to product development of the Company which may not be successful or to the marketing authorizations granted by competent authorities or,

more generally, any factors that may affect marketing capacity of the products developed by AB Science, as well as those developed or identified in the public documents filed by AB Science with the Autorité des Marchés Financiers (AMF), including those listed in the Chapter 4 "Risk Factors" of AB Science reference document filed with the AMF on November 22, 2016, under the number R. 16-078. AB Science disclaims any obligation or undertaking to update the forward-looking information and statements, subject to the applicable regulations, in particular articles 223-1 et seq. of the AMF General Regulations.

For additional information, please contact:

AB Science

Financial Communication & Media Relations

investors@ab-science.com